
Large vs Small Scale Studies

Overview

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Small Scale Studies

Small Scale Studies

- Provide a test of whether treatments are successful under controlled conditions
- Test hypotheses derived from a theoretical model
- Test mechanisms responsible for treatment effects

The Major Challenge

- Behavioral clinical trials:

Getting people to change their behavior

Question:

- Under what conditions can you get people to change their behavior?

Key Features of Small-Scale Studies

Key Features-1

- Standardized treatment protocols
 - Treatment manuals
 - Therapist training
 - Audiotaping of sessions
 - Ongoing supervision

Key Features-2

- Carefully screened subjects
 - standard diagnostic criteria
 - trained examiners
 - elimination of comorbid conditions
 - special exclusions

Key Features-3

- Randomization
 - benefits of randomization
 - randomization by concealment

Key Features-4

- Control conditions:
 - Historical control
 - Waiting list controls
 - Standard care control**
 - Comparison condition (e.g. education) control**
 - Attention placebo control

Key Features-5

- Blinding
 - blinding of evaluators
 - blinding of subjects
 - blinding of providers

Key Features-6

- Outcome measures
 - One primary outcome
 - Secondary outcomes from multiple domains
 - Repeated measurements
 - Follow-up

Challenges of Small-Scale Studies

Nature of Treatment Setting

- Optimal condition
- Research lab setting
- Often removed from clinical setting
- Therapists hired for study
- Participants don't pay for treatment

Generalizability

- Samples often specialized
- Standardized treatments may not be representative
- Therapists may be more (less skilled) than typical clinicians

Threats to Validity

- Differential refusal
- Differential dropout
- Baseline differences despite randomization
- Analysis of treatment completers only
- Intensity of treatment

Large Scale Studies

Large Scale Studies

- Provide a test of whether treatments are successful under controlled conditions (multi-site efficacy study)

or

- Provide a test of whether treatments are successful under real-world conditions (multi-site effectiveness study)

Benefits of Large Scale Studies

- 1. Enhance recruitment
- 2. Complete trial sooner
- 3. Rigorous monitoring via coordinating center

Key Features

Key Features-1

- Coordinating Center
 - Initial development of protocols
 - data collection
 - randomization
 - Ongoing monitoring of data collection
 - Interim data analyses
 - Interaction with DSMB
 - Interaction with funding agency

Key Features-2

- Standardization and monitoring of treatment protocols by coordinating center
 - Treatment manuals
 - Therapist training
 - Audiotaping of sessions
 - Ongoing supervision

Key Features-3

- Carefully screened subjects
 - standard diagnostic criteria
 - trained examiners
 - elimination of comorbid conditions
 - special exclusions

Key Features-4

- Control condition:
 - Standard care control
 - or
 - Comparison condition control

Key Features-5

- Outcome measures
 - One primary outcome
 - Possibly secondary outcomes
 - Repeated measurements
 - Follow-up

Challenges

Nature of Treatment Setting

- May not be optimal conditions to deliver treatment
- Can be clinical or research lab setting
- Therapists backgrounds vary across sites

Generalizability

- Samples often less specialized
- Treatments may be more representative
- Therapists may be less skilled and trained than in smaller studies

Threats to Validity

- Intensity of treatment

Discussion